Developing a therapeutic candidate for Canavan disease using induced pluripotent stem cell

Grant Award Details

Developing a therapeutic candidate for Canavan disease using induced pluripotent stem cell

Grant Type: Early Translational II

Grant Number: TR2-01832

Project Objective: to derive iPSC-derived NSC's from canavan disease patients (3) , correct by gene therapy and test efficacy in mouse CD model by year 3.

Investigator:

Name:	Yanhong Shi
Institution:	City of Hope, Beckman Research Institute
Type:	PI

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Name:	Oliver Brustle
Institution:	Universität Bonn
Type:	Partner-PI

Disease Focus: Genetic Disorder, Neurological Disorders, Pediatrics

Collaborative Funder: Germany

Human Stem Cell Use: iPS Cell

Cell Line Generation: iPS Cell

Award Value: $1,835,983

Status: Closed

Progress Reports

Reporting Period: Year 1

View Report

Reporting Period: Year 2

View Report

Reporting Period: Year 3

View Report

Reporting Period: Year 4/NCE Annual

View Report

Grant Application Details

Application Title: Developing a therapeutic candidate for Canavan disease using induced pluripotent stem cell

Public Abstract: Canavan disease is a devastating disease of infants which affects their neural development and leads to mental retardation and early death. It occurs in 1 in 6,400 persons in the U.S. and there is no treatment so far. We propose to generate genetically-repaired and patient-specific stem cells (called iPSCs) from patients' skin cells, and then coax these stem cells into specific types of corrective neural precursors using methods established in our laboratories in order to develop a therapeutic candidate for this disease. By use of a mouse model of Canavan disease, we will determine the ability of these genetically corrected cells to successfully treat the disease. These results will form the basis for an eventual clinical trial in humans, and if successful, would be the first treatment for this terrible disease.

There are many families affected by this disease, and other diseases similar to it. Results from this work could have applications to this and other similar genetic diseases. Through the proposed research, maybe no parents will have to watch their child suffer and die as a result of these dreadful diseases in one day. What a wonderful day that would be!

Statement of Benefit to California: It is estimated that California has ~12% of all cases of Canavan disease in the U.S. Besides the tremendous emotional and physical pain that this disease inflicts on families, it produces in California a medical and fiscal burden that is larger than any other states. Thus, there is a real need to develop a strategy of treatment for this disease. Stem cells provide great hope for the treatment of a variety of human diseases that affect the citizens of California. Combination of gene therapy and iPSC technology will enable the development of therapeutic candidates of human genetic diseases via the creation of genetically-corrected patient-specific iPSCs. Our proposal aims to establish a therapeutic development candidate for Canavan disease, a devastating neurodegenerative disease that leads to mental retardation and early death. The generation of genetically-repaired and patient-specific iPSC lines will represent great potential not only for California health care patients but also for pharmaceutical and biotechnology industries in California. Moreover, California is a strong leader in pre-clinical and clinical research developments. To maintain this position, we need to create patient-specific stem cells as autologous therapeutic candidates, in order to overcome the challenges of immune rejection faced by today's cell therapy field. This proposal addresses the very issue by generating "disease-corrected" and patient-specific iPSCs as a therapeutic candidate with the potential to create safer and more effective cell replacement therapies.

pluripotent-stem